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Dockets Management Staff
Food and Drug Administration
5630 Fishers Lane, Room 1061
Rockville, MD 20852

RE: Public Meeting on the Recommendations for Reauthorization of the Prescription Drug User Fee Act (PDUFA); Request for Comments [FDA-2026-N-8163]

Submitted electronically via regulations.gov

The National Health Council (NHC) appreciates the opportunity to submit comments to the U.S. Food and Drug Administration (FDA) on the proposed Prescription Drug User Fee Act (PDUFA) Performance Goals and Procedures for fiscal years 2028 through 2032 (PDUFA VIII). We also appreciate FDA's continued consultation with patient and consumer stakeholders throughout the reauthorization process, including the public stakeholder meetings held during negotiations and the September 16, 2026, public meeting on the proposed recommendations.¹

The NHC unites nearly 200 national organizations—including leading patient groups, research institutions, providers, caregivers, and businesses across the health care sector—to drive patient-centered health policy. Representing 200+ million Americans with chronic diseases and disabilities, the NHC strengthens its members' collective influence to expand access to quality, affordable, and equitable health care. The NHC fosters collaboration to shape policies that reflect the needs of patients.

The proposed commitment letter reflects the conclusion of an extensive FDA-industry negotiation and stakeholder consultation process and provides a constructive framework for the human drug review program during fiscal years 2028 through 2032. The NHC recognizes the substantial work required to reach agreement on a package that maintains core review performance goals while addressing first-cycle review, meeting management, rare disease development, patient-focused drug development (PFDD), real-world evidence (RWE), regulatory science, postmarket safety, manufacturing facility assessment, staffing, information technology, and financial stewardship. These commitments to improve regulatory predictability, scientific capacity, communication, and the quality and relevance of evidence supports the outcomes that matter to patients: timely access to safe and effective therapies and the

¹ U.S. Food and Drug Administration, "Reauthorization of the Prescription Drug User Fee Act; Public Meeting; Request for Comments," 91 Fed. Reg. 52702 (August 14, 2026), <https://www.federalregister.gov/documents/2026/08/14/2026-16650/reauthorization-of-the-prescription-drug-user-fee-act-public-meeting-request-for-comments>.

information needed to make informed treatment decisions.² The NHC's overall view of the proposed agreement is positive. At this stage in the reauthorization process, our recommendations are intended to focus primarily on supporting effective implementation of commitments already included in the agreement. They also identify areas where existing reporting and evaluation mechanisms can strengthen accountability and shared learning. The NHC also encourages continued patient-community engagement as FDA develops guidance, training, case studies, evaluation methods, and other supporting activities. Consistent with the approach the NHC took in commenting on the MDUFA VI draft commitment letter, we do not seek to reopen the negotiated performance framework or create duplicative requirements where the agreement already provides a workable mechanism for implementation.³

As FDA implements these commitments, PDUFA VIII's success should be measured by more than review speed or process efficiency. During FDA's stakeholder consultations, patient and consumer representatives repeatedly emphasized the importance of public health outcomes, evidentiary rigor, transparency, and the quality of regulatory review alongside timeliness. The NHC agrees that efficiency is valuable when it supports high-quality regulatory decision-making. FDA should therefore assess whether faster or more predictable processes are accompanied by consistent scientific review, timely identification and resolution of material safety and efficacy questions, and sufficient capacity to perform the work rigorously.⁴

Increased predictability itself has value for both patients and developers. Sponsors make development, manufacturing, staffing, and investment decisions over many years, while patients and patient organizations depend on a regulatory system capable of evaluating increasingly complex therapies without avoidable delay. Patient-centered implementation should therefore reinforce, rather than compete with, the agreement's goals of scientific rigor, timely communication, consistent review, and operational sustainability.

Where the NHC recommends additional transparency, patient engagement, or methodological attention, we favor approaches that use existing PDUFA structures, remain fit for purpose, protect confidential commercial information, preserve FDA's independent scientific judgment, and avoid unnecessary administrative burden. These priorities are also consistent with themes raised by industry stakeholders, including the importance of a strong, appropriately staffed FDA, predictable and science-based review, clear communication, financial sustainability, transparency, and efficient

² U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures, Fiscal Years 2028 Through 2032" (2026), <https://www.fda.gov/industry/prescription-drug-user-fee-amendments/pdufa-viii-fiscal-years-2028-2032>.

³ National Health Council, "NHC Comments on Medical Device User Fee Amendments Performance Goals and Procedures for Fiscal Years 2028 Through 2032," August 6, 2026, <https://nationalhealthcouncil.org/letters-comments/nhc-comments-on-medical-device-user-fee-amendments-performance-goals-and-procedures-for-fiscal-years-2028-through-2032/>.

⁴ U.S. Food and Drug Administration, "Prescription Drug User Fee Act (PDUFA) Reauthorization: Stakeholder Meeting with FDA," April 20, 2026, <https://www.fda.gov/media/192546/download>.

implementation of negotiated commitments.^{5,6} The recommendations below build on the NHC's August 2025 comments at the opening of the PDUFA VIII reauthorization process and on patient-community priorities raised during FDA's stakeholder consultation series. Those comments emphasized a well-resourced FDA, integration of patient experience data, voluntary patient-centered outcome development, RWE, review consistency, regulatory science, and the importance of moving successful pilots into durable practice to avoid an increasingly fragmented regulatory landscape. The proposed commitment letter addresses many of those priorities directly. Our comments therefore focus on how implementation can maximize their value to patients and developers while maintaining the balance reflected in the negotiated agreement.⁷

Summary of Recommendations

The NHC encourages FDA to build on the negotiated PDUFA VIII framework by:

- Fully implementing and adequately resourcing the PFDD commitments, including the planned case studies, public meeting, training, and continued review-document statements, and using those activities to clarify when and how patient experience data inform regulatory decision-making.
- Advancing voluntary, fit-for-purpose use of patient-centered outcome and impact measures through existing PFDD, rare disease, endpoint-development, and regulatory-science mechanisms while preserving flexibility in methods and evidence generation.
- Implementing the rare disease and Rare Disease Endpoint Advancement (RDEA) commitments in ways that preserve scientific judgment, strengthen consistency across review teams, facilitate early engagement on endpoints and development strategy, and support meaningful contributions from patients and patient organizations to cross-cutting scientific work.
- Maintaining dedicated FDA expertise, reviewer training, sponsor interaction, and public reporting for RWE, with attention to data fitness, patient-relevant outcomes, representativeness, longitudinal experience, and clear communication about when and how RWE informs regulatory decisions.
- Using the first-cycle review and formal-meeting assessments to identify recurring opportunities to improve the clarity, timing, and consistency of FDA-sponsor communication and reduce avoidable complete responses, review-cycle extensions, and rework without weakening standards for safety, effectiveness, product quality, or evidentiary rigor.

⁵ Biotechnology Innovation Organization, "BIO Supports PDUFA VIII Commitment Letter and Urges Timely Reauthorization," August 13, 2026, <https://www.bio.org/press-release/bio-supports-pdufa-viii-commitment-letter-and-urges-timely-reauthorization>.

⁶ Pharmaceutical Research and Manufacturers of America, "Comments on Reauthorization of the Prescription Drug User Fee Act," August 14, 2025, https://cdn.aglty.io/phrma/global/resources/import/pdfs/FDA-2025-N-0816-0056_PhRMA%20comments%20on%20Reauth.pdf.

⁷ National Health Council, "NHC Comments on Public Meeting on Reauthorization of the Prescription Drug User Fee Act," August 14, 2025, <https://nationalhealthcouncil.org/letters-comments/nhc-comments-on-public-meeting-on-reauthorization-of-the-prescription-drug-user-fee-act/>.

- Implementing the Chemistry, Manufacturing, and Controls (CMC) facility lifecycle program as a risk-based mechanism for earlier identification and resolution of facility-readiness and inspection issues, while evaluating whether the new engagement tools reduce facility-related complete responses and avoidable delays in patient access.
- Strengthening Sentinel and Risk Evaluation and Mitigation Strategies (REMS) assessment capabilities through fit-for-purpose postmarket evidence strategies that consider patients' treatment experiences, meaningful outcomes, and burden when relevant to the safety question under review.
- Maintaining sufficient FDA staffing, retention, training, and specialized expertise across CDER, CBER, and supporting offices and using existing hiring and performance reporting to assess FDA's capacity to deliver PDUFA VIII commitments consistently.
- Implementing data, technology, bioinformatics, and artificial intelligence investments in ways that improve review quality, consistency, and efficiency while maintaining appropriate validation, scientific oversight, security, and accountability.
- Addressing study feasibility, participation burden, and representativeness through early, fit-for-purpose planning, recognizing that practical barriers can affect recruitment, retention, data completeness, and the relevance of evidence to the populations expected to use a therapy.
- Building transparency and accountability through PDUFA VIII's existing reporting, case studies, public meetings, and evaluations, while protecting confidential commercial information and avoiding duplicative reporting structures.
- Complementing FDA-industry Sentinel interactions with regular public-facing information on Sentinel 3.0 priorities, methodological developments, implementation progress, and major safety learnings, while protecting confidential information and data restricted by use agreements.
- Monitoring the effects of proposed fee-structure changes on FDA resources, smaller developers, and incentives for rare and ultra-rare disease development, and using existing financial reporting to identify unintended consequences without creating duplicative reporting requirements.
- Evaluating PDUFA performance through a balanced set of measures that weights timeliness and process efficiency alongside review quality, scientific capacity, resolution of material evidentiary issues, and appropriate postmarket follow-through, rather than using speed alone to define success.
- Continuing structured engagement with patient and consumer stakeholders throughout implementation using existing public meetings, workshops, listening sessions, requests for comment, and other proportionate mechanisms to bring patient perspectives into cross-cutting policy and scientific work.

The Proposed Agreement Represents Constructive and Meaningful Progress

The NHC appreciates the substantial work undertaken by FDA and the biopharmaceutical industry to reach a negotiated commitment package. The proposal preserves PDUFA's core purposes by supporting FDA's scientific and operational capacity, maintaining predictable review performance, and facilitating timely patient access to safe and effective medicines. The proposed agreement retains the principal

review timelines for NDAs, BLAs, resubmissions, and efficacy supplements while pairing those goals with enhanced communication, independent program assessments, expanded meeting-management tools, and investments in scientific capabilities that reflect the increasing complexity of modern drug development.⁸ Clearer review expectations, earlier identification of substantive issues, timely communication, and greater consistency across review teams can reduce avoidable delays and unnecessary rework while supporting stronger development programs. For sponsors, predictable interactions can improve planning and permit evidentiary, statistical, manufacturing, or operational concerns to be addressed while development plans remain adaptable. For patients, the corresponding value is a regulatory process that identifies critical questions early, supports evidence generation for informed decision-making, and preserves FDA's standards for safety and effectiveness.

The proposed agreement also recognizes that performance cannot be measured only by whether a goal date was technically met. The independent first-cycle assessment will examine complete responses, major amendments, review-clock extensions, communications, labeling interactions, and pivotal-protocol discussions across PDUFA VII and VIII. It is designed to identify patterns and best practices across CDER and CBER and to produce actionable recommendations aimed at reducing avoidable complete responses, missed goal dates, and review extensions for applications that are ultimately approvable. The NHC supports this approach because meeting aggregate timing goals does not eliminate avoidable uncertainty when significant issues are identified late, communicated inconsistently, or handled differently across review divisions without a scientific reason.⁹

As is appropriate for a negotiated user fee agreement, many of the most detailed provisions address FDA-industry interactions, staffing, review operations, inspections, finances, and technology. The NHC seeks to ensure that these mechanisms produce clear benefits for patients. Aggregate reporting and well-selected case examples can help stakeholders understand whether earlier communication reduced unnecessary rework, whether specialized expertise improved consistency, whether patient experience data were meaningfully evaluated, whether RWE contributed to regulatory decisions, or whether facility lifecycle engagement prevented an additional review cycle. These forms of transparency can strengthen both patient confidence and sponsor understanding without exposing confidential commercial information.

The NHC's August 2025 comments similarly emphasized the importance of moving successful pilots into durable, predictable practice rather than allowing disconnected initiatives to proliferate. Programs should be expanded or sustained when evidence demonstrates value, while those that do not demonstrate measurable benefit or scalability should be reconsidered. The draft agreement reflects that by including the integration of complex innovative designs into standard review practice, the planned transition of RDEA and model-informed drug development opportunities into established

⁸ U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," secs. I.A–I.B.

⁹ U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," sec. I.C.

Type C meeting pathways, and the use of independent assessments to determine whether new meeting and facility mechanisms are working as intended. The NHC encourages FDA to maintain that implementation discipline throughout the five-year cycle.¹⁰

Review Performance Goals Should Be Understood as a Floor for Predictability, Not the Sole Measure of Program Success

The NHC supports continuation of the core review performance goals for original NDAs and BLAs, resubmissions, efficacy supplements, and manufacturing supplements. Under the proposed agreement, FDA would continue to review and act on 90 percent of standard NME NDAs and original BLAs within 10 months of the 60-day filing date and 90 percent of priority NME NDAs and original BLAs within six months of that date, with established timelines for other submission categories. Stable performance give FDA, sponsors, investigators, patients, and other stakeholders a common basis for planning and assessing program performance over time.¹¹

The NHC recommends that FDA and stakeholders interpret these goals in the context of the broader review process. An application can technically meet its action date while still experiencing avoidable inefficiency when material questions are raised late, advice changes without sufficient explanation, or communication problems increase the likelihood of an additional cycle. Conversely, the agreement's major-amendment rules appropriately preserve flexibility for review extensions, generally limiting them to circumstances in which new information could address outstanding deficiencies and permit approval in the current cycle.

A review program that meets patient needs combines predictable timelines with sufficiently early identification of issues, clear explanations of deficiencies, and the scientific flexibility needed to consider new information that could resolve those deficiencies. The first-cycle assessment, meeting enhancements, pivotal-protocol prioritization, facility lifecycle program, and staffing commitments should work together to improve review quality and predictability. FDA's public performance reporting can reinforce this broader view by continuing to report whether formal goals are met while using findings from independent assessments can explain recurring causes of extensions, complete responses, or other process friction. These findings can help distinguish legitimate scientific complexity from remediable operational problems and provide context that timing metrics alone cannot capture.

The NHC also encourages FDA to the efficiency of the review process from the quality of regulatory decisions. No single metric can capture the quality of an individual approval or complete response, and approval rates themselves are not an appropriate proxy. FDA should draw on independent assessments and existing reporting to examine whether gains in timeliness are accompanied by consistent application of scientific

¹⁰ National Health Council, "NHC Comments on Public Meeting on Reauthorization of the Prescription Drug User Fee Act."

¹¹ U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," secs. I.A–I.B.

standards, timely identification of material evidentiary and safety issues, continuity of specialized expertise, appropriate resolution of product-quality concerns, and completion of necessary postmarket evidence generation. This broader address concerns raised during FDA's April stakeholder consultation that PDUFA performance measures should provide greater visibility into public-health outcomes and not focus on efficiency alone.¹²

Patient-Focused Drug Development Is a Core Strength of the Agreement

The NHC strongly supports continuation of the PFDD program and the agreement's explicit focus on shared learning about the use of patient experience data. The draft defines PFDD as a systematic approach to collecting and using robust and meaningful patient and caregiver experiences, perspectives, needs, and priorities to inform drug development and regulatory decision-making. FDA commits to continue outreach on how patient experience data can be submitted with an NDA or BLA and considered in regulatory decision-making, including labeling, as appropriate. It would also publish case studies at least twice by the end of FY 2032, convene a public meeting by the end of FY 2030, and continue internal and external training. FDA would also continue publishing brief public statements in review documents about patient experience data submitted and reviewed with an application.¹³

These commitments directly address a gap between recognizing the value of patient experience data and understanding when those data are relevant and fit for purpose for a specific regulatory question, which is a challenge consistently identified by patient organizations and developers. The PFDD program should therefore help sponsors, patient organizations, researchers, and reviewers understand what makes these data useful, how methodological limitations are weighed, and how patient-derived evidence is considered alongside clinical, statistical, biomarker, natural-history, and other evidence in an application. FDA's 2023 request for information on methodological challenges related to patient experience data specifically sought input on barriers to regulatory use, fit-for-purpose clinical outcome assessments, endpoint selection, patient preference studies, and submission of patient experience data, underscoring the need for the shared learning contemplated in PDUFA VIII.¹⁴

The planned case studies are especially valuable because they can translate general methodological principles into concrete examples of how patient experience data apply to specific regulatory decisions. The NHC encourages FDA to use them to describe, where feasible, the regulatory question under consideration, the type and source of patient experience data, and the stage of development at which the information was

¹² U.S. Food and Drug Administration, "Prescription Drug User Fee Act (PDUFA) Reauthorization: Stakeholder Meeting with FDA," April 20, 2026, <https://www.fda.gov/media/192546/download>.

¹³ U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," sec. I.L.5.f.

¹⁴ U.S. Food and Drug Administration, "Methodological Challenges Related to Patient Experience Data; Request for Information and Comments," 88 Fed. Reg. 27521 (May 2, 2023), <https://www.federalregister.gov/documents/2023/05/02/2023-09265/methodological-challenges-related-to-patient-experience-data-request-for-information-and-comments>.

generated or discussed. The case studies should also illustrate the methodological considerations that affected whether and how the data informed the decision. The agreement appropriately contemplates examples in which patient experience data were used and examples in which they were not used. The latter may be just as informative when FDA can explain why a measure, study, or dataset did not answer the regulatory question. Transparent discussion of limitations can help sponsors and patient organizations improve future work without turning a successful example into an informal requirement for other programs.¹⁵ The NHC's own collection of patient-focused medical product development case examples likewise illustrates how practical examples can make patient engagement and patient-derived evidence more concrete for developers and patient organizations.¹⁶

The NHC also encourages FDA to use PFDD training and internal consultation to promote greater consistency across review teams. The weight given to patient experience data may appropriately vary with the disease, endpoint, intended population, measurement properties, missing data, and the data's role in the overall evidence package. Consistency therefore does not mean identical outcomes across applications. It means that sponsors receive reasonably predictable explanations of the factors FDA considers, reviewers have access to relevant expertise in patient-focused methods, and departures from established practice are explained in the context of the scientific circumstances of the individual program.

Patient organizations can support this work without participating in confidential FDA-sponsor review interactions. Disease-specific organizations can provide knowledge of symptoms, functional limitations, treatment burdens, risk tolerance, heterogeneity, natural history, and the practical meaning of clinical change. Likewise, cross-cutting organizations can identify methodological themes that extend across conditions. Caregivers may provide essential insight when disease or treatment limits patients' ability to report their own experience. FDA can gather these perspectives through the planned public meeting, and other appropriate opportunities, including workshops, guidance development, and listening sessions.

Implementation should also recognize that meaningful patient engagement does not require direct engagement at every stage of every development program. Existing patient experience data, preference studies, registries, natural-history studies, published qualitative research, or prior externally led PFDD work may already provide substantial insight. A fit-for-purpose approach allows FDA and sponsors to determine whether additional engagement would materially improve the evidence plan or help answer the regulatory question, preserving both patient-community capacity and development efficiency.

¹⁵ U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," sec. I.L.5.f.i–iii.

¹⁶ National Health Council, "Patient-Focused Medical Product Development Case Examples," accessed September 21, 2026, <https://nationalhealthcouncil.org/additional-resources/patient-focused-medical-product-case-examples/>.

Patient-Centered Outcomes Should Advance Through Voluntary, Fit-for-Purpose Approaches

The NHC's 2025 PDUFA comments recommended that FDA support development of a voluntary Patient-Centered Core Outcome Set (PC-COS) approach aligned with patient-centered core impact set principles. The purpose of such an approach is to improve consistency in identifying the disease and treatment impacts that matter most to patients, so that study outcomes and measures reflect those priorities. The proposed commitment letter does not create a distinct PC-COS program, but its PFDD, rare disease, RDEA, regulatory-science, and reviewer-training provisions provide opportunities to advance this objective.¹⁷

Greater consistency in the domains assessed across studies can improve comparability and help researchers build on prior findings, particularly when several products or development programs target the same disease. Depending on the condition, patient-relevant domains may include symptoms, functioning, independence, cognitive or emotional effects, ability to work or attend school, treatment burden, caregiver burden, tolerability, and the ability to participate in ordinary activities. Traditional clinical measures remain essential, but they may not fully capture whether a therapy produces a change that patients recognize and value in daily life. Core outcome set methodology similarly demonstrates how agreement on what should be measured can improve comparability while leaving subsequent decisions about how to measure those domains to the needs of a particular study.¹⁸

The NHC strongly supports preserving methodological flexibility. Identifying core impacts should not prescribe a single instrument, endpoint hierarchy, statistical model, trial design, or evidentiary configuration. The appropriate measures depend on the disease, stage of development, intended indication, mechanism of action, measurement feasibility, age and functional characteristics of the population, and the role the endpoint is intended to play in the regulatory decision. FDA's implementation activities should therefore encourage early identification of meaningful concepts and appropriate measures without creating the expectation that every sponsor use the same endpoint. Patient-reported outcomes should not displace other clinical measures or biomarkers when those measures better address the regulatory question.

The NHC's Blueprint for Developing Patient-Centered Core Impact Sets provides a patient-led process for identifying and prioritizing disease and treatment impacts that can inform later measurement decisions. FDA can draw on this and other established methods through workshops, case studies, technical assistance, and public discussions. Where several sponsors or research groups are confronting similar endpoint challenges, shared methodological work can reduce duplication and create

¹⁷ National Health Council, "NHC Comments on Public Meeting on Reauthorization of the Prescription Drug User Fee Act."

¹⁸ Paula R. Williamson et al., "The COMET Handbook: Version 1.0," *Trials* 18, suppl. 3 (2017): 280, <https://doi.org/10.1186/s13063-017-1978-4>.

resources for future development programs, including smaller companies and rare disease programs with fewer resources.¹⁹

Breakthrough Therapy and Early Surrogate Endpoint Consultation Can Reduce Development Uncertainty

The NHC supports continued investment in the Breakthrough Therapy program and the agreement's early consultation pathway for new surrogate endpoints. This pathway allows sponsors to seek advice on a biomarker that has not previously served as the primary basis for approval in the proposed context of use. PDUFA VIII would retain resources for intensive FDA guidance and senior-management involvement in breakthrough programs and would provide a Type C meeting pathway for early discussion of novel surrogate endpoints. These mechanisms are particularly relevant for serious and life-threatening diseases in which development time, uncertainty about endpoint acceptability, and limited opportunities for conventional evidence generation can directly affect whether a promising therapy advances.²⁰

Early surrogate-endpoint consultation can create value even when FDA ultimately concludes that additional evidence is necessary. A sponsor that understands at an early stage what is known about the biomarker, what uncertainty remains, and what evidence would be needed to establish its relationship to clinical benefit can address those questions in development planning before a pivotal study begins. Patient input can also clarify the clinical meaning and importance of the outcome the biomarker is intended to predict, particularly when the disease has substantial morbidity that is difficult to measure directly within a feasible trial period.

The NHC encourages FDA to use guidance, case examples, and cross-program learning to make the surrogate-endpoint consultation pathway more predictable while preserving case-specific scientific judgment. The biological relationship between a biomarker and clinical benefit, the strength of available evidence, the disease context, treatment effect, residual uncertainty, and feasibility of confirmatory evidence can differ materially across programs. Transparency should therefore explain the reasoning and evidentiary considerations behind a decision rather than suggesting that use of a particular biomarker in one context automatically establishes suitability in another.

The Breakthrough Therapy program similarly depends on timely, coordinated advice from relevant FDA disciplines and sustained support throughout development. Staffing transitions and growing workloads should not interrupt access to the expertise and senior-management involvement associated with the designation. FDA's broader workforce and meeting-management commitments should support the program by ensuring that designation translates into meaningful access to expertise throughout

¹⁹ National Health Council, A Blueprint for Developing Patient-Centered Core Impact Sets (Washington, DC: National Health Council, 2022), <https://nationalhealthcouncil.org/a-blueprint-for-developing-patient-centered-core-impact-sets-pc-cis/>.

²⁰ U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," secs. I.L.1–I.L.2.

development rather than simply an expedited label attached to a conventional interaction model.

Rare Disease Commitments Should Preserve Flexibility While Strengthening Predictability

The rare disease provisions are among the most important patient-centered elements of PDUFA VIII. FDA would continue to integrate rare disease expertise into CDER and CBER review teams, train reviewers on the challenges associated with rare disease applications and strategies for addressing them, engage externally with industry and patient groups, and convene Rare Disease Innovation, Science, and Exploration (RISE) workshops to address scientific and development barriers. The agreement also commits FDA to reporting on training, participation of rare disease staff in review programs, stakeholder engagement, use of expedited programs, and regulatory flexibility in rare disease approvals.²¹ These provisions align with priorities identified by rare disease organizations. The National Organization for Rare Disorders (NORD) in its PDUFA VIII recommendations emphasized strengthening the Rare Disease Innovation Hub, patient input, endpoint development, incorporating lessons from successful pilots into routine practice, reviewer training, innovation for limited populations, and a well-resourced and agile FDA. The agreement addresses many of those priorities through the rare disease staffing model, RISE workshops, RDEA continuation, cross-center work, and broader commitments to regulatory-science tools and review consistency.²²

The NHC supports an approach to rare disease regulation that maintains scientific rigor while tailoring development approaches to the disease and regulatory question. Small populations, slowly progressive conditions, heterogeneity, limited natural-history information, irreversible morbidity, and ethical or practical constraints on conventional randomized trials can make standard development pathways difficult or impossible to execute. PDUFA VIII appropriately focuses on FDA expertise, interaction, endpoint development, innovative designs, biomarkers, natural history, statistical approaches, and use of expedited programs rather than prescribing a single solution.²³ FDA's LEADER 3D stakeholder analysis documented many of these same challenges, including limited natural-history data, uncertainty around novel endpoints and biomarkers, small and heterogeneous populations, and highlighted the need for clearer education and regulatory expectations.²⁴

²¹ U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," sec. I.L.3.

²² National Organization for Rare Disorders, "NORD Comments on Reauthorization of PDUFA VIII," August 14, 2025, <https://rarediseases.org/wp-content/uploads/2025/08/NORD-Comments-on-Reauthorization-of-the-Prescription-Drug-User-Fee-Act-8.14.2025.pdf>.

²³ U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," sec. I.L.3.

²⁴ U.S. Food and Drug Administration, LEADER 3D: Learning and Education to Advance and Empower Rare Disease Drug Developers: Public Report of External Stakeholder Analysis (March 2024), <https://www.fda.gov/media/176557/download>.

This flexibility also applies to how sponsors demonstrate substantial evidence of effectiveness. The statutory standard can be satisfied through different configurations of sufficiently persuasive evidence, and flexibility should not be understood as a lower evidentiary standard. FDA should continue to consider the totality and context of evidence permitted under law, including the appropriate use of one rigorous pivotal investigation together with confirmatory evidence when scientifically justified. The agency remains responsible for determining whether the evidence establishes effectiveness for the proposed indication. For rare diseases in particular, predictable application of this flexibility can be as important as the availability of flexibility in principle.

The NHC encourages FDA to use reviewer training, rare disease staff participation, RISE workshops, RDEA experience, guidance, public examples and early written feedback to clarify how factors underlying those judgments clearer and promote more consistent application across review teams. When novel designs, external controls, natural-history data, Bayesian methods, biomarkers, surrogate endpoints, or other nontraditional approaches are used successfully, FDA should explain the scientific conditions that supported their use and any limitations. When such approaches are not accepted, explanations of evidentiary gaps can likewise improve future development. Public examples should be sufficiently general to protect confidential sponsor information and avoid creating rigid precedents across diseases with materially different characteristics. Regulatory flexibility is most useful when sponsors and patient communities understand the scientific considerations that inform its use. FDA need not establish rigid criteria for when an alternative design, external control, surrogate endpoint, or other nontraditional approach will be acceptable; doing so could constrain the case-specific judgment that rare disease development often requires. This balance also responds to stakeholder concerns that flexibility in principle provides limited value when developers cannot anticipate the evidentiary considerations that will guide review.²⁵

The NHC also supports meaningful patient participation in rare disease implementation. In many rare conditions, patient organizations have built natural-history datasets, registries, endpoint-development programs, and research networks because the traditional evidence base was limited. Their expertise can help FDA and sponsors understand disease heterogeneity, disease progression, clinically meaningful change, feasibility of study procedures, and the relative importance of potential benefits and risks. That expertise is particularly valuable in cross-cutting workshops and methodological initiatives, where lessons can inform multiple development programs.

RDEA Can Move Endpoint Development from Pilot Activity Toward Durable Practice

The NHC strongly supports continuation of the RDEA program. The agreement recognizes that limited regulatory precedent, small trial populations, and incomplete understanding of disease natural history complicate the selection and development of

²⁵ U.S. Food and Drug Administration, "Prescription Drug User Fee Act (PDUFA) Reauthorization: Stakeholder Meeting with FDA," February 2, 2026, <https://www.fda.gov/media/191325/download>.

efficacy endpoints for rare disease therapies. During FY 2028 and 2029, FDA would continue the RDEA pilot with a limited number of proposals, before transitioning to Type C-RDEA meetings within the formal PDUFA meeting framework in FY 2030 through 2032.²⁶

This transition is consistent with the NHC's support for integrating successful user fee pilots into established review practice. Endpoint development often requires iterative scientific discussion, and Type C meeting structure can make RDEA more predictable and scalable while preserving access to specialized expertise. The NHC encourages FDA to evaluate the pilot-to-Type C transition by meeting volume alongside whether sponsors receive timely access to appropriate experts, whether advice is sufficiently clear to inform development decisions, and whether methodological lessons are shared across review teams.

The agreement's planned RDEA workshops and opportunities to share public examples can extend learning beyond individual programs. Novel endpoints may not transfer directly across diseases, but lessons about their meaningfulness, measurement properties, sensitivity to change, relationship to clinical benefit, and suitability for particular trial designs may apply more broadly. Patient organizations should remain active participants in those discussions because their expertise can help distinguish statistically detectable changes from changes that patients experience as meaningful.

The NHC further encourages coordination between RDEA and the broader PFDD program so that endpoint-development work draws on patient experience while avoiding duplicative engagement. In some programs, established patient-focused measures or natural-history datasets may already provide sufficient information about relevant concepts; where gaps remain, targeted additional engagement may be warranted. The objective should be a clear, efficient pathway from understanding what matters to patients to selecting or developing a measure capable of supporting the intended regulatory use.

Real-World Evidence Commitments Can Improve Both Regulatory Learning and Development Predictability

The NHC supports the agreement's continued investment in real-world data (RWD) and RWE. PDUFA VIII would maintain dedicated resources, train relevant subject matter experts and review-team members, provide opportunities for sponsors to request RWE expertise through formal meetings, update guidance as experience develops, and continue reporting of aggregate, anonymized information on RWE submissions to CDER and CBER. The reporting would identify application type, data source, study design, and regulatory request and, where appropriate, summarize characteristics of RWD and RWE that contributed to a determination of substantial evidence of effectiveness for a new indication.²⁷

²⁶ U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," sec. I.L.3.a.

²⁷ U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," sec. I.L.5.g.

This framework appropriately emphasizes both scientific rigor and predictability. The regulatory usefulness of RWE depends on the research question, data provenance and quality, completeness, validation, outcome ascertainment, confounding, missing data, follow-up, and the relevance of the study population to the regulatory decision. A data source or study design may be highly informative for one purpose and poorly suited to another. Public reporting should therefore explain why a data source and design were fit for the regulatory question, without implying that one source is always preferred.

The patient community can contribute to this fit-for-purpose assessment. Many NHC member organizations represent people who live with chronic diseases and disabilities for years or decades. Outcomes and burdens that matter to these populations may emerge outside clinical encounters, change over long periods, or be incompletely represented in claims and electronic health records. Patient organizations can help identify meaningful follow-up periods, outcomes that are not routinely captured, reasons for treatment discontinuation or switching, practical barriers to adherence, and differences among subgroups that may affect interpretation.

The NHC's patient-centered RWE recommendations describe how patient input can inform research questions, outcome and data-source selection, study design and conduct, interpretation, and dissemination. Patient-centeredness in this context does not mean that patients direct every methodological decision. The aim is to keep evidence generation connected to the questions, outcomes, and burdens relevant to the population expected to use the therapy while preserving appropriate methodological judgment. FDA can advance this principle through public methodological work and stakeholder engagement without requiring a separate patient-engagement process for every RWE submission.²⁸ An exploratory analysis applying the NHC's Patient Experience Mapping Toolbox to real-world data studies further illustrates how patient centricity can be assessed across research questions, data sources, study conduct, interpretation, and dissemination.²⁹

The NHC also supports aggregate annual reporting and links to public review documents that explain where RWE is being used, which methods supported regulatory decisions, and the context for individual approvals. This approach can improve shared learning without compromising confidential commercial information and may be particularly useful to smaller sponsors and academic investigators with fewer opportunities to learn from repeated FDA interactions. Where RWE is used as confirmatory evidence alongside a pivotal investigation, as external or supplemental evidence in rare disease development, or to support postapproval requirements, FDA should assess whether the overall evidence is sufficiently reliable and persuasive for the

²⁸ Elisabeth Oehrlein, Jennifer Graff, Jason Harris, and Eleanor Perfetto, Patient-Centered Real-World Evidence: Methods Recommendations from an Evidence-Based Consensus Process (Washington, DC: National Health Council, May 2021), <https://nationalhealthcouncil.org/wp-content/uploads/2021/06/NHC-Patient-Centered-Real-World-Evidence-White-Paper-1.pdf>.

²⁹ Elisabeth Oehrlein, Mehmet Burcu, Silke Schoch, and Laura Gressler, "Enhancing Patient Centricity of Real-World Data Research: An Exploratory Analysis Using the Patient Experience Mapping Toolbox," *Value in Health* 26, no. 1 (January 2023): 10–17, <https://doi.org/10.1016/j.jval.2022.10.002>.

specific regulatory decision. Greater transparency regarding successful uses and limitations can make these assessments more predictable while preserving FDA's case-specific scientific judgment.

Structured Benefit-Risk Assessment Should Make Patient-Relevant Reasoning More Visible

The NHC supports PDUFA VIII's continuation of structured benefit-risk assessment in NDA and BLA review. FDA currently incorporates the Benefit-Risk Framework into reviewer training, processes, and templates. Under the commitment letter, FDA would continue its implementation and explore additional opportunities to enhance use and communication of benefit-risk assessments. This work is central to patient-focused regulation because it weighs benefits and risks together in light of the seriousness of the condition, available therapies, magnitude and durability of benefit, nature and uncertainty of risks, and the evidence supporting each conclusion.³⁰

Patient experience can provide important context within that framework. Clinical endpoints alone may not capture how people with serious chronic or rare conditions weigh functional improvement, symptom relief, treatment convenience, durability, uncertainty, or particular adverse effects. Patient preference studies and other forms of patient experience data can help characterize these tradeoffs when collected and interpreted appropriately, while FDA retains responsibility for determining how the information fits within the total evidence.

The NHC encourages FDA to continue improving the public communication of benefit-risk reasoning in review documents and other decision materials. A structured framework is most useful to patients and clinicians when it explains the principal benefits, risks, uncertainties, and contextual factors that drove the decision rather than functioning primarily as an internal template. Clear communication can also help patient organizations identify where additional evidence or education is needed after approval. Public communication should provide enough detail for patients, clinicians, and other stakeholders to understand FDA's reasoning while protecting confidential information. After a decision, FDA can use existing review documentation and patient-facing materials to explain scientific uncertainty and acknowledge differences in how patients may value tradeoffs.

Regulatory Science Tools Should Move Toward Consistent Review Practice Without Becoming Prescriptive

PDUFA VIII continues substantial investment in regulatory science, including structured benefit-risk assessment, complex innovative designs, digital health technologies, biomarker qualification, model-informed drug development, PFDD, RWE, and bioinformatics. The agreement also commits FDA to ongoing reviewer training across these tools and to a public meeting by the end of FY 2028 on best practices for

³⁰ U.S. Food and Drug Administration, Benefit-Risk Assessment for New Drug and Biological Products: Guidance for Industry (October 2023), <https://www.fda.gov/regulatory-information/search-fda-guidance-documents/benefit-risk-assessment-new-drug-and-biological-products>.

communicating how regulatory science approaches are used in drug development and regulatory decision-making. The NHC supports this integrated approach because innovative methods are most useful when review teams understand their strengths, sponsors can access appropriate expertise, and experience gained in one program can inform future work.³¹

The transition of the Complex Innovative Design program into standard review practice can make scientifically appropriate designs more widely accessible. FDA would maintain capacity and subject matter expertise for adaptive, Bayesian, and other novel designs. Sponsors could seek advice through existing formal meeting pathways or special protocol assessment where eligible. This approach can reduce the artificial distinction between conventional and innovative methods by making scientifically appropriate designs part of normal regulatory practice rather than exceptional tools available only through a small pilot.³²

The NHC encourages FDA to evaluate regulatory-science implementation by the quality and consistency of scientific interaction rather than by utilization alone. A higher number of meetings or projects can demonstrate activity but does not establish that sponsors received timely, actionable advice or that reviewers applied the method consistently. Public examples should therefore describe the problem the tool was intended to address, the strengths and limitations identified by FDA, and the type of development or review decision it informed. Explaining the context and limitations can make FDA's reasoning clearer while reducing the risk that case studies become rigid precedents.

The agreement's public meeting on communication of regulatory science tools provides a useful forum for patient and consumer participation. Patients may not need to engage in the technical details of every modeling or statistical method, but they can help identify what information matters to the public when innovative methods materially affect the evidence supporting a therapy. For example, patient communities may benefit from clear explanations of how an external control was constructed, how a digital measure relates to daily functioning, or what uncertainty remains when model-informed methods reduce the need for a conventional study component.

Digital Health Technologies and AI Should Support Better Evidence Without Creating New Barriers

The NHC supports PDUFA VIII's continued investment in digital health technologies (DHTs), including those enabled by artificial intelligence (AI). The agreement recognizes their potential to generate information outside traditional clinical visits and commits FDA to maintain resources for consistent review of DHTs, coordinate across review centers as appropriate, and use workshops, demonstration projects, guidance, and review experience to support shared learning. DHTs can reduce travel and site burden, permit more continuous measurement, and capture symptoms or functional changes that

³¹ U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," secs. I.L.5–I.L.7.

³² U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," sec. I.L.5.b.

periodic clinical visits may miss.³³ Their value, however, depends on whether the technology and resulting data are fit for the intended population and purpose. Not every digital study is expected to represent every population equally, but limitations in who can use a tool may influence the interpretation or generalizability of the evidence related to a specific research question. Device access, broadband connectivity, smartphone ownership, digital literacy, disability accommodations, language, caregiver support, and the burden of repeated measurement can all affect who participates and the completeness of resulting data.

The NHC encourages FDA to use existing DHT implementation activities to help sponsors identify and address these barriers early through solutions suited to the study and population. Such solutions may include providing devices, offering alternative data-collection options, testing accessibility, reducing measurement frequency, and providing local or remote support. Where barriers remain, sponsors should explain the limitations and their implications for the evidence. A proportionate approach can improve data quality and participation without turning accessibility considerations into a rigid checklist.

FDA's technology modernization, including its use of AI-enabled analytic tools, should maintain appropriate human oversight, validation, documentation, and accountability. These investments should help reviewers manage complex data and workflows while preserving scientific judgment. Aggregate public reporting can describe modernization activities and whether they improve timeliness, consistency, or review quality.

Model-Informed Drug Development Should Become More Accessible While Preserving Scientific Flexibility

The NHC supports expansion of the Model-Informed Drug Development (MIDD) meeting program and the planned transition toward Type C-MIDD meetings considered on a rolling basis. The draft would increase the number of eligible proposals accepted beginning in FY 2028 and work toward eliminating the quarterly cadence and cap by the end of FY 2030, with meetings led by quantitative medicine leadership in partnership with relevant clinical, statistical, and other disciplines. This can make access to specialized modeling expertise more predictable and integrate MIDD more fully into routine development.³⁴

When models and underlying data are fit for purpose, MIDD can improve dose selection, support extrapolation and development in populations that are difficult to study and reduce unnecessary studies. For patients, these improvements can mean less meaningful participation burden and reduced exposure to ineffective or poorly tolerated regimens.

The NHC encourages FDA to pair expanded access with continued reviewer training and public examples so that sponsors understand the questions MIDD can appropriately address and the limitations that may require additional evidence. As with

³³ U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," sec. I.L.5.c.

³⁴ U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," sec. I.L.5.e.

other regulatory-science tools, these activities should build broad scientific capability while preserving flexibility to choose methods suited to each program. Public communication should therefore explain, where feasible, the development problem the model addressed, the implications for study design and evidence generation, and any remaining uncertainty.

Pivotal Protocol Prioritization and Special Protocol Assessment Can Improve Development Decisions Before They Become Difficult to Change

The NHC supports PDUFA VIII's emphasis on earlier review of pivotal protocols and continuation of the Special Protocol Assessment (SPA) process. The proposed agreement calls for sponsors to identify pivotal protocols, planned study start dates, and critical questions in the submission cover letters. FDA would prioritize review consistent with relevant Manual of Policies and Procedures (MAPP) and Standard Operating Policies and Procedures (SOPP) timelines, update those procedures by the end of FY 2028, and train review staff on pivotal-protocol prioritization. For eligible protocols, the SPA process continues to provide a formal mechanism through which sponsors can seek agreement on whether design, execution, and analysis are adequate to meet identified scientific and regulatory requirements.³⁵

Timely FDA feedback on critical protocol questions can help sponsors resolve avoidable uncertainty about endpoints, analysis, study populations and other design elements before enrollment begins. These mechanisms protect patients from protocol problems that may waste time, increase participant burden, and undermine research opportunities. These risks particularly acute in rare diseases and other small populations, where repeating a trial may be infeasible.

The NHC encourages FDA to implement pivotal-protocol prioritization in a manner that is operationally clear to sponsors and supported by sufficient reviewer capacity. The commitment will have limited value if a protocol is labeled as pivotal but substantive questions remain unanswered until after the study start date. FDA's first-cycle assessment appropriately includes evaluation of the elapsed time between submission of pivotal protocols and FDA responses, the timing of statistical analysis plans, and the relationship between pivotal-protocol review practices and later review outcomes. Those findings should inform training and internal process improvement. SPA and pivotal-protocol review should also preserve appropriate scientific flexibility. Agreement on a protocol is valuable because it reduces uncertainty about design and analysis, but development necessarily evolves as new safety information, scientific knowledge, or public health concerns emerge. The NHC encourages FDA to continue explaining clearly when later information requires departure from earlier advice and should distinguish circumstances in which the underlying science changed from those in which inconsistent interpretation across review personnel created avoidable uncertainty.

³⁵ U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," secs. I.I-I.J.

First-Cycle Review Assessments Should Focus on Avoidable Process and Communication Barriers

The independent assessment of first-cycle review is one of the most consequential operational provisions in PDUFA VIII. The third party will evaluate first-cycle outcomes, the timing and basis of complete responses and major amendments, adherence to key process milestones, labeling communications, pivotal-protocol interactions, and the timing and substance of FDA-sponsor communications across review divisions. The NHC strongly supports this opportunity to identify why additional review cycles occur and where changes in process or communication could prevent avoidable delays.³⁶

The assessment should examine both the substantive unresolved safety, effectiveness, manufacturing, or evidentiary issues behind complete responses and extensions and whether process, timing, coordination, or communication may have contributed to an avoidable additional cycle. The NHC supports identifying and addressing issues as early as possible while maintaining evidentiary standards and ensuring deficiencies are resolved before approval.

The agreement's planned review of communication timing is especially important. Early, clear communication may allow a sponsor to resolve a deficiency within the existing cycle; raising the same issue later may leave insufficient time to respond. Aggregate analysis can help determine whether certain patterns reoccur across divisions or application types and whether specific communication practices are associated with better first-cycle outcomes.

The NHC encourages FDA and the independent evaluator to produce interim and final reports that are useful to both industry and the public. Reports should describe categories of issues and process patterns, identify practices associated with timely resolution, and explain when observed variation appears scientifically justified. Findings should inform guidance, MAPPs, SOPPs, reviewer training, and program management while respecting application-specific scientific judgments. Patient and consumer stakeholders should be able to inform the assessment through the planned public comment opportunities on its statement of work and interim report. Their perspective may be especially useful in identifying downstream patient consequences of avoidable delay and in evaluating whether performance measures reflect patient priorities. FDA and the independent evaluator would retain responsibility for the technical assessment of individual reviews.

Formal Meeting Enhancements Should Improve Clarity, Consistency, and Access to the Right Expertise

PDUFA VIII makes several meaningful changes to formal meetings, including mechanisms for multi-divisional meetings, clearer procedures for choosing between face-to-face meetings and written responses, preliminary-response timing goals for certain meeting types, opportunities for clarification after meetings or written responses,

³⁶ U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," sec. I.C.

and an independent assessment of selected enhancements. These provisions can help sponsors obtain timely expertise and resolve questions before uncertainty affects later development decisions.³⁷

The NHC supports efficient use of written responses while preserving opportunities for real-time discussion when the issues warrant it. Complex development programs may involve clinical, statistical, pharmacology, manufacturing, device, digital, or rare disease issues that cannot be resolved effectively in isolation. Multi-divisional meetings can be especially useful when a sponsor would otherwise receive sequential or potentially inconsistent advice from different parts of the Agency.

The clarification process is also valuable because ambiguity after a formal meeting can become a source of avoidable downstream disagreement. Meeting minutes summarize discussions, and ambiguity about whether a sponsor correctly understood FDA's advice can create significant downstream risk. Allowing focused clarifying questions within a defined period can improve shared understanding without converting the process into a second substantive meeting or creating an open-ended obligation for FDA. The NHC encourages consistent implementation across review divisions so that sponsors can reasonably predict when a clarification request is within scope. The independent assessment should evaluate whether these enhancements improve the timeliness and usefulness of communication, including whether sponsors receive access to relevant disciplines, whether format decisions are reasonably consistent, and whether clarifying questions reduce later disagreement or rework. Public reporting on aggregate findings can also help FDA identify where staffing or internal coordination, rather than the formal meeting rules themselves, is the principal constraint.

Postmarketing Requirements Should Be Planned Early and Designed Around the Question That Needs to Be Answered

The proposed agreement continues established processes for communicating anticipated postmarketing requirements (PMRs) before the PDUFA action goal date and for discussing study purpose, design elements, population, and timelines. Early communication to enable sufficient planning is particularly important for studies involving long follow-up, rare outcomes, small populations, pregnancy, comparative safety, or evidence that conventional trials may be poorly suited to collect.³⁸ The NHC encourages FDA and sponsors to use these pre-approval discussions to define the question that the PMR is intended to answer and to select a study design and data source that are proportionate to that question. Study requirements that do not reflect clinical practice or patient experience can contribute to slow enrollment, incomplete follow-up, or evidence that is difficult to interpret. Early planning can help determine whether a randomized study, registry, Sentinel analysis, claims or electronic health record study, prospective observational design, or other approach is most appropriate.

³⁷ U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," sec. I.K.

³⁸ U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," sec. I.D.

Where the PMR will depend materially on patient participation, patient organizations may be able to inform feasibility, meaningful outcomes, acceptable follow-up burden, and retention challenges through cross-cutting or disease-specific engagement. Such input can help identify practical barriers before a study is finalized while preserving does FDA's scientific judgment or sponsor responsibility for study design. The value of premarket flexibility is also linked to reliable postmarket follow-through when important uncertainty remains at approval. The NHC encourages FDA to continue improving the timeliness and transparency of PMRs, including clear communication regarding the purpose and status of required studies and timely completion of confirmatory evidence where applicable. Patients, particularly those with substantial unmet needs, should be able to understand what is known at approval, what remains uncertain, and how required studies will address that uncertainty. FDA's January stakeholder consultation similarly highlighted concerns about long or opaque PMR timelines and the need for clearer scientific justification and adherence to postmarket commitments.³⁹

Sentinel and REMS Modernization Should Strengthen Safety Oversight While Reducing Unnecessary Burden

The NHC supports continued investment in FDA's drug safety system, including modernization of REMS assessment and optimization of the Sentinel Initiative. Under PDUFA VIII, user fees would support REMS assessment planning and review as well as maintenance of Sentinel data infrastructure, implementation of Sentinel 3.0, training of review staff, and exploration of a voluntary sponsor concept brief for a possible non-interventional PMR related to a safety issue.⁴⁰ Sentinel is an important component of a learning regulatory system because it can help FDA evaluate safety questions using large distributed datasets without requiring a new prospective study for every question. The NHC encourages continued attention to the populations represented in underlying data, the clinical detail available for the outcome of interest, and the limitations created by missing variables or changing patterns of care. When Sentinel analyses materially inform regulatory action, accessible public communication can help patients and clinicians understand the question evaluated, the principal findings, and important limitations.

Because Sentinel supports regulatory decisions involving products used by large patient populations, the NHC encourages FDA to complement the technical FDA-industry interactions contemplated by PDUFA VIII with regular public summaries of major Sentinel 3.0 priorities, methodological developments, implementation progress, and lessons relevant to patient safety. These materials need not disclose confidential information or data restricted by data-use agreements. Where feasible, the NHC encourages FDA to continue exploring mechanisms that allow qualified independent researchers and public stakeholders to understand and assess the methods and findings used to address important safety questions. Patient and consumer

³⁹ U.S. Food and Drug Administration, "Prescription Drug User Fee Act (PDUFA) Reauthorization: Stakeholder Meeting with FDA," January 9, 2026, <https://www.fda.gov/media/191193/download>.

⁴⁰ U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," sec. I.M.

stakeholders raised similar concerns during FDA's consultations, including limited public access to postmarket safety data and the need for greater transparency regarding Sentinel analyses.⁴¹

REMS modernization should similarly focus on whether the strategy achieves its risk-management objective while minimizing burden that does not materially contribute to safe use. Patients, caregivers, prescribers, pharmacies, and health systems can experience REMS requirements differently, and burdens may affect access or adherence when processes are difficult to navigate. FDA's assessment planning can benefit from measures that capture both risk-mitigation performance and the practical effects of the program, as well as from incorporating patient input when it can help identify unintended barriers. The NHC appreciates that the agreement maintains a defined performance goal for FDA review of REMS assessment methods and protocols. Timely feedback can allow sponsors to implement assessment strategies consistently and can reduce later disagreement about whether the evidence adequately measures program performance.

Cell and Gene Therapy Commitments Require Sustained Expertise, Manufacturing Capacity, and Long-Term Learning

The NHC strongly supports continuation and strengthening of FDA's Cell and Gene Therapy Program. The agreement would sustain specialized CBER staffing, training, policy development, external outreach, stakeholder education, postmarket safety work, and collaboration on manufacturing, testing, bioinformatics, artificial intelligence, standards, and new approaches to evidence generation. It also directs continued attention to rare diseases, small populations, novel trial designs, surrogate endpoints, natural histories, RWE, and methods for capturing postapproval safety and efficacy.⁴² These therapies illustrate why PDUFA must support more than application review timelines. Development can depend on highly specialized manufacturing and assay capabilities, complex comparability questions, long-term follow-up, limited patient populations, and rapidly evolving scientific knowledge. Regulatory predictability therefore depends on continuity of expertise and opportunities for early scientific discussion as much as it depends on meeting the final review goal date.

Patient organizations can add particular value in cell and gene therapy development because benefits, risks, and uncertainties may be experienced over long periods. Affected communities often have substantial knowledge of disease natural history and treatment burden. Cross-cutting engagement can help identify outcomes that remain meaningful over time, practical considerations for long-term follow-up, and communication needs when uncertainty persists after approval. The NHC encourages FDA to continue integrating patient perspectives through the stakeholder outreach mechanisms already contemplated by the program rather than creating separate engagement requirements for individual applications. The agreement also appropriately

⁴¹ U.S. Food and Drug Administration, "Prescription Drug User Fee Act (PDUFA) Reauthorization: Stakeholder Meeting with FDA," January 9, 2026, <https://www.fda.gov/media/191193/download>.

⁴² U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," sec. I.O.

recognizes the potential to leverage prior knowledge and public knowledge across therapeutic contexts when legally and scientifically appropriate. Efficient use of relevant prior knowledge can reduce unnecessary duplication, but its use should remain transparent enough for sponsors to understand what information can reasonably be bridged or relied upon and what new evidence is needed for the specific product.

The CMC Facility Lifecycle Program Can Address Avoidable Manufacturing-Related Delays

Manufacturing and facility issues can prevent approval of an otherwise clinically approvable application, and PDUFA VIII includes a significant new CMC facility lifecycle program intended to identify and address these issues earlier. The agreement calls for guidance on manufacturing-facility readiness, permits a CMC facility pre-submission meeting, creates additional engagement mechanisms before, during, and after review, and provides for an assessment of the program's impact on facility-related complete responses.⁴³ The NHC supports this approach because earlier communication can benefit both product quality and timely access. A facility that does not meet applicable requirements must be remediated, and a PDUFA goal date cannot substitute for adequate manufacturing assurance. However, deficiencies that can be identified and corrected earlier should not remain unresolved simply because relevant information or communication arrives too late in the review cycle. Readiness guidance and pre-submission discussion can help applicants and facilities understand expectations before an inspection occurs.

Implementation should remain risk based and should account for the complexity of global supply chains and the interdependence of manufacturing operations. The agreement contemplates discussion of supply-chain relationships, lessons from prior inspections, facility readiness, and quality information in advance of submission. This can give FDA a stronger basis for inspection planning and allow sponsors to focus remediation resources on issues most likely to affect approvability. The planned independent assessment should examine whether the program changes outcomes in practice: whether significant deficiencies are identified earlier, whether communications are sufficiently actionable, whether opportunities to resolve issues within the current cycle are used when appropriate, and whether facility-related complete responses decline without compromising product quality. Aggregate reporting should also distinguish cases in which an additional cycle remained necessary because deficiencies were substantive from cases in which earlier engagement could have changed the timeline.

Study Design, Participation Burden, and Representativeness Should Be Addressed Early

Patient-centered drug development requires attention to whether studies are feasible for the populations expected to participate. Protocols may require repeated travel, specialty-center visits, invasive procedures, lengthy assessments, caregiver

⁴³ U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," sec. I.N.

involvement, digital technology, or time away from work and family responsibilities. Some of these requirements are scientifically necessary, but avoidable burdens can affect recruitment, retention, protocol adherence, data completeness, and the representativeness of the resulting evidence. The NHC encourages FDA and sponsors to consider these questions early, when protocol elements remain modifiable. FDA's guidance on decentralized trial elements recognizes that remote visits, local health care providers, direct-to-participant approaches, and other tools may be appropriate in some studies, while emphasizing that the design must remain suitable for the investigational product, population, and scientific question. Patient organizations can help identify which procedures create the greatest practical burden and where flexibility could improve participation without reducing scientific rigor.⁴⁴

Representativeness should likewise be evaluated in relation to the intended population and the clinical questions for which differences may matter. Age, sex, race and ethnicity, disability, disease severity, comorbidities, geography, language, socioeconomic circumstances, and access to specialty care or technology can affect participation and, in some contexts, treatment response, safety, adherence, or measurement. FDA's diversity action plan provides a framework for prospective consideration of underrepresented populations, while PDUFA's early interaction and PFDD mechanisms can support more practical planning.⁴⁵ The NHC does not support a rigid enrollment formula that disregards disease epidemiology, feasibility, small populations, or the scientific purpose of a study. Rather, sponsors should be encouraged to identify populations relevant to the regulatory question, plan recruitment and retention accordingly, and describe meaningful limitations when full representation is not feasible. Patient organizations can also help distinguish barriers created by study design from broader barriers such as referral patterns, insurance coverage, geography, language, transportation, caregiving, or lack of specialty centers.

Older adults warrant particular attention because comorbidities, polypharmacy, functional limitations, transportation, and caregiver needs can affect both study participation and treatment experience. NHC member organizations focused on healthy aging have long highlighted the importance of aligning regulatory policy with an aging population, increasing older-adult participation, and reducing avoidable barriers to clinical trial participation. Fit-for-purpose inclusion and analysis can improve the relevance of evidence for the population likely to use a therapy without requiring every study to answer every geriatric question.⁴⁶

⁴⁴ U.S. Food and Drug Administration, Conducting Clinical Trials with Decentralized Elements: Guidance for Industry, Investigators, and Other Interested Parties (September 2024), <https://www.fda.gov/regulatory-information/search-fda-guidance-documents/conducting-clinical-trials-decentralized-elements>.

⁴⁵ U.S. Food and Drug Administration, Diversity Action Plans to Improve Enrollment of Participants from Underrepresented Populations in Clinical Studies: Draft Guidance for Industry (June 2024), <https://www.fda.gov/regulatory-information/search-fda-guidance-documents/diversity-action-plans-improve-enrollment-participants-underrepresented-populations-clinical-studies>.

⁴⁶ Alliance for Aging Research, "Alliance Submits Comments to U.S. Food & Drug Administration Regarding the Inclusion of Older Adults in Cancer Clinical Trials," May 4, 2020,

Reviewer Capacity, Retention, Training, and Consistency Remain Foundational

The NHC strongly supports the draft agreement's emphasis on rebuilding and maintaining the scientific workforce required for the human drug review program. The commitment letter acknowledges high attrition among technical and scientific experts during PDUFA VII and commits FDA to quarterly updates on PDUFA hiring, including designated positions associated with model-informed drug development, rare diseases, the facility lifecycle program, and Sentinel.⁴⁷ Staffing adequacy cannot be assessed solely by the total number of positions filled. Continuity, institutional knowledge, effective onboarding, supervisory capacity, mentoring, cross-office consultation, and retention of specialized expertise all affect whether additional resources translate into consistent regulatory performance. Frequent reviewer transitions can disrupt development programs and require sponsors to revisit previously discussed questions, while stable review teams can provide more coherent scientific advice over time.

This is especially important in areas such as rare disease, cell and gene therapy, quantitative medicine, RWE, complex trial design, bioinformatics, manufacturing, and digital technologies, where expertise is specialized and methods continue to evolve. The agreement's reviewer-training commitments across regulatory science tools are therefore an essential complement to hiring. Training can promote a common understanding of available methods and encourage appropriate use while still allowing scientifically justified differences across products and disease areas.⁴⁸

The NHC encourages FDA to use existing staffing and performance reporting to provide a clear picture of whether capacity is sufficient to implement PDUFA VIII commitments. Aggregate reporting can include progress in priority hiring areas, retention trends where available, major training initiatives, and whether staffing constraints are materially affecting meeting availability, review timelines, or access to subject matter expertise. The objective should be operational accountability, not disclosure of information about individual employees or review teams.

The NHC also continues to emphasize that PDUFA user fees supplement rather than replace the need for robust federal appropriations. A stable human drug review program depends on broader FDA infrastructure, scientific policy, management, inspection, information technology, and public health functions that extend beyond the activities directly supported by user fees. Financial sustainability therefore requires both responsible stewardship of user-fee resources and sufficient appropriated funding to maintain the Agency's institutional capacity.

<https://www.agingresearch.org/blog/alliance-submits-comments-to-u-s-food-drug-administration-regarding-the-inclusion-of-older-adults-in-cancer-clinical-trials/>.

⁴⁷ U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," sec. III.

⁴⁸ U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," sec. I.L.6.

Information Technology, Bioinformatics, and AI Modernization Should Improve the Review Environment

PDUFA VIII includes substantial commitments related to information technology, data exchange, bioinformatics, cloud infrastructure, and emerging technologies. FDA would continue quarterly discussions with regulated industry on modernization priorities and implementation, including system consolidation and harmonization, electronic submission infrastructure, cloud technology, artificial intelligence, and other tools that affect the regulatory submission and review process.⁴⁹ The NHC supports modernization because outdated or fragmented systems can create inefficiency for both FDA and sponsors and can make it harder for reviewers to analyze increasingly complex datasets. The bioinformatics commitments are particularly important as genomic, multi-omic, computational, and other high-dimensional data become more common. FDA's plan to maintain specialized expertise, software, storage, computing capacity, and international work on data standards can help ensure that technical limitations do not become avoidable barriers to scientific review.⁵⁰

Implementation of AI within FDA's own review environment should be evaluated based on whether it improves quality, consistency, speed, or reviewer capacity while maintaining appropriate human oversight. Automated tools may be useful for document management, data extraction, coding support, signal detection, or other tasks, but the scientific and regulatory judgments required for drug review should remain the responsibility of qualified FDA personnel. Validation, security, auditability, and management of model changes should therefore be part of implementation from the outset.

The quarterly FDA-industry meetings provide an appropriate forum for detailed operational feedback because sponsors experience submission systems directly and can identify recurring technical problems. Patient and consumer stakeholders do not need to participate in every technical meeting, but FDA's public reporting should clearly explain the overall direction of modernization, particularly when changes materially affect review performance, data integrity, transparency, or the FDA's ability to evaluate patient-centered evidence.

Transparency and Accountability Should Build on Existing PDUFA Reporting

The NHC supports the agreement's substantial transparency and accountability provisions. PDUFA VIII would continue annual performance and financial reports, require a five-year financial plan and annual updates, expand financial detail, provide technical staff meetings on fee-setting and finances, publish quarterly hiring information, and establish several independent assessments with public reports and opportunities for comment. Together, these mechanisms can help stakeholders understand whether user-fee resources are aligned with workload and whether negotiated enhancements

⁴⁹ U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," sec. IV.A.

⁵⁰ U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," sec. I.L.8.

are producing the intended operational results.⁵¹ The NHC encourages FDA to build on this reporting framework rather than create separate reporting systems for each patient-centered initiative. Existing reports, implementation updates, case studies, public meetings, and program evaluations can be organized so that stakeholders can follow progress in PFDD, RWE, rare disease, regulatory science, staffing, Sentinel, facility lifecycle work, and other major commitments over time. The value of transparency lies in making performance understandable, not in maximizing the volume of reporting.

Where feasible, the NHC encourages FDA to distinguish measures of activity from measures of impact. The number of staff trained, meetings held, workshops convened, or case studies published can demonstrate implementation, but these metrics do not by themselves establish whether those activities improved development planning or regulatory practice. Examples of impact could describe how early communication resolved a recurring issue, how PFDD information affected an endpoint or labeling decision, how RWE contributed to an approval or postmarket requirement, how a RISE workshop informed guidance or training, or how facility lifecycle engagement prevented an avoidable additional cycle. This type of transparency can benefit developers as well as patients because practical examples clarify FDA's reasoning and help sponsors determine when an approach may be useful. The NHC recognizes that application-specific communications, trade secrets, confidential commercial information, and unpublished development strategies must be protected. Aggregate, anonymized, and carefully selected examples can support shared learning without compromising those protections.

The first-cycle assessment also provides an opportunity to provide greater detail on complete responses and major review extensions. Public reporting could categorize significant causes at an aggregate level—such as clinical, statistical, product-quality, inspection, labeling, or other substantive issues—and, where sample size and confidentiality protections permit, identify meaningful patterns across broad application or therapeutic categories. FDA's February stakeholder consultation reflected interest in more granular information about first-cycle outcomes and complete responses. Carefully designed aggregate reporting could provide that context without disclosing confidential application correspondence or implying that a complete response is inappropriate when substantive deficiencies remain.⁵² The enterprise performance assessment provides another important accountability mechanism. The contractor will evaluate productivity baselines, quantitative and qualitative measures of efficiency, workload, staffing, timeliness and quality. It will also assess whether any realized efficiencies should affect PDUFA revenue amounts. The NHC encourages a balanced framework that does not treat apparent reductions in processing time or staffing inputs as efficiency gains if they are associated with reduced review quality, loss of scientific capacity, deferred work, or greater burdens elsewhere in the system. Efficiency should

⁵¹ U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," secs. II–VI.

⁵² U.S. Food and Drug Administration, "Prescription Drug User Fee Act (PDUFA) Reauthorization: Stakeholder Meeting with FDA," February 2, 2026, <https://www.fda.gov/media/191325/download>.

be understood in relation to the full set of PDUFA responsibilities and the quality of regulatory output.⁵³

Fee Structure Changes Should Preserve FDA Capacity and Rare Disease Incentives

The broader PDUFA VIII recommendations also include proposed changes to the statutory fee structure that are not contained in the performance-goals letter itself. FDA proposes a 50 percent reduction in the application fee for qualifying applications that include clinical data from at least one Phase 1 trial anchored in the United States and initiated after October 1, 2027. It also proposes a fee equal to 50 percent of the full application fee for the first supplement seeking a non-orphan indication for an application that previously received the orphan application-fee exception. The proposals would limit the orphan program-fee exemption to products approved only for orphan indications; and restrict of the small-business waiver to U.S.-based applicants.⁵⁴

The NHC recognizes the policy objectives underlying these proposals, including supporting domestic clinical development, addressing circumstances in which a product moves beyond an exclusively orphan market, and maintaining a sustainable user-fee program. Implementation and subsequent reporting should nevertheless monitor whether the changes have unintended effects on smaller developers, development strategies for rare and ultra-rare diseases, or FDA's available resources. During FDA's January stakeholder consultation, several patient and consumer participants expressed reservations about changes to orphan fee exceptions and exemptions and emphasized preserving incentives for rare disease development. The NHC therefore encourages implementation that maintains a strong rare-disease development ecosystem while ensuring that fee treatment remains reasonable as products expand into non-orphan indications.⁵⁵

These questions are well suited to the financial transparency mechanisms already contemplated by PDUFA VIII. The five-year financial plan, annual financial reports, fee-setting notices, and enterprise performance assessment can help FDA, user-fee payers, patient organizations, and Congress understand whether the revised structure is operating as intended. The NHC does not recommend a separate reporting regime; rather, existing reports should clearly explain how major fee changes affect revenue, staffing, workload, and the sustainability of scientific and patient-engagement capabilities.

⁵³ U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," sec. II.C.

⁵⁴ U.S. Food and Drug Administration, "Reauthorization of the Prescription Drug User Fee Act; Public Meeting; Request for Comments," 91 Fed. Reg. 52702, 52707 (August 14, 2026), <https://www.govinfo.gov/content/pkg/FR-2026-08-14/pdf/2026-16650.pdf>.

⁵⁵ U.S. Food and Drug Administration, "Prescription Drug User Fee Act (PDUFA) Reauthorization: Stakeholder Meeting with FDA," January 9, 2026, <https://www.fda.gov/media/191193/download>.

Financial Sustainability and Stewardship Are Patient-Centered Issues

The NHC views the financial sustainability of PDUFA as foundational to patient-centered implementation. Adequate and predictable resources allow FDA to retain expert staff, provide timely scientific advice, evaluate complex evidence, conduct inspections, modernize information technology, support postmarket safety, and meet negotiated review commitments. Responsible financial stewardship and transparent reporting are equally important because user-fee payers and the public need confidence that resources are aligned with authorized activities and program needs. The proposed five-year financial plan and enhanced reporting on shared services, payroll, operating expenses, reserves, and other financial categories can improve understanding of how resources support the human drug review program. The technical staff meetings with regulated industry provide a useful mechanism for clarifying fee-setting and financial questions without burdening the commitment letter with excessive operational detail. Public meeting minutes can extend that learning to other stakeholders.⁵⁶

The NHC supports continued attention to the relationship between workload, staffing, and resources. Biomedical innovation can change workload in ways that are not captured by application counts alone because the complexity of data, number of formal interactions, specialized scientific questions, postmarket responsibilities, and manufacturing issues may all affect staff time. Resource planning should therefore remain responsive to both volume and complexity so that FDA is not forced to choose between meeting timeline goals and maintaining the scientific work necessary for high-quality review.

Implementation Should Include Continued Patient-Community Engagement

The NHC appreciates the opportunities FDA has provided for patient and consumer input throughout PDUFA VIII, including the stakeholder consultation series, public meetings, and opportunities for written commitment. FDA's May 2026 stakeholder meeting confirmed the conclusion of negotiations and highlighted continued opportunities for public engagement through the clearance process, formal comment periods, and public meetings. Continued engagement will remain important because many of the agreement's patient-relevant commitments will be shaped through subsequent guidance, workshops, case studies, training, reporting methods, and program assessments. The NHC encourages FDA to use established and proportionate engagement mechanisms, including public meetings, listening sessions, workshops, requests for information, advisory activities, and opportunities to comment on guidance or implementation materials. A separate consultation structure is unlikely to be necessary for every PDUFA initiative. Engagement is most valuable when the question is sufficiently defined for patients and patient organizations to contribute expertise that FDA cannot obtain from operational metrics or sponsor interactions alone.⁵⁷

⁵⁶ U.S. Food and Drug Administration, "PDUFA Reauthorization Performance Goals and Procedures," sec. II.B.

⁵⁷ U.S. Food and Drug Administration, "PDUFA VIII Stakeholder Meeting with FDA," May 20, 2026, <https://www.fda.gov/media/193003/download>.

Depending on the issue, relevant perspectives may come from individual patients, family caregivers, disease-specific organizations, organizations representing people with disabilities, and cross-cutting patient coalitions. Patient organizations can identify knowledgeable participants, explain variation within a condition, provide context on natural history and treatment burden, help interpret patient-generated evidence, and disseminate information back to affected communities. These contributions complement the scientific, regulatory, manufacturing, and development expertise provided by FDA, industry, clinicians, and researchers.

Practical conditions affect who can participate and how effectively they can contribute. Advance notice, accessible and plain-language background materials, virtual participation when appropriate, sufficient preparation time, and reasonable attention to disability, caregiving, travel, and technology barriers can broaden participation and improve the quality of input. These practices are particularly important for smaller organizations and individual patients who may not have dedicated regulatory staff. The NHC is prepared to support FDA's implementation efforts by helping identify patient and caregiver experts, convening relevant communities, providing cross-disease methodological perspectives, and disseminating information about PDUFA initiatives. Continued multi-stakeholder engagement can reinforce confidence in the program while preserving the negotiated FDA-industry mechanisms that are central to user-fee implementation.

Conclusion

The proposed PDUFA VIII commitment letter reflects extensive negotiation and stakeholder consultation and provides a strong framework for the next user fee cycle. The NHC supports the agreement's continued investments in FDA capacity, predictable review, effective FDA-sponsor communication, patient-focused drug development, rare disease expertise, RWE, regulatory science, postmarket safety, manufacturing and facility engagement, specialized staffing, and data and technology modernization. Taken together, these commitments can strengthen both the quality and efficiency of drug development and review while supporting timely patient access to safe and effective therapies.

The NHC's recommendations are intended primarily to support implementation of the negotiated framework rather than to reopen the agreement. In many instances, the patient-centered potential of PDUFA VIII can be advanced through the case studies, guidance, training, workshops, public reporting, stakeholder engagement, and independent assessments already contemplated by the commitment letter. These mechanisms can make regulatory reasoning more understandable, help sponsors identify evidentiary and operational issues earlier, and improve the relevance of evidence without creating unnecessary new burdens or reducing the flexibility needed for innovation.

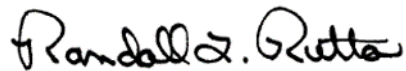
Effective implementation will require continued collaboration among FDA, the biopharmaceutical industry, patients, caregivers, clinicians, researchers, and other stakeholders. Industry brings essential scientific, technical, manufacturing, and development expertise and provides user-fee resources that are central to FDA's review capacity. Patients and caregivers contribute direct knowledge of disease, treatment

burden, meaningful benefit, risk tolerance, feasibility, and the uncertainties that matter in real-world decisions. FDA's independent scientific judgment and regulatory oversight provide the foundation for integrating those perspectives in a manner that protects patients while supporting innovation.

For patients, the success of PDUFA VIII will ultimately be reflected in whether development and review proceed efficiently and predictably and whether evidence addresses benefits, risks, outcomes, and uncertainties that matter to affected populations. Success will also depend on whether FDA maintains the expertise and consistency necessary for rigorous oversight, and whether learning from innovative approaches is translated into better development programs over time. The proposed agreement provides a strong foundation for advancing those objectives.

Please do not hesitate to contact Jennifer Dexter, Senior Vice President, Policy & External Affairs, at jdexter@nhcouncil.org, or Shion Chang, Assistant Vice President, Policy & Regulatory Affairs, at schang@nhcouncil.org, if you or your staff would like to discuss these comments in greater detail. The NHC appreciates FDA's consideration of these comments and looks forward to continued engagement as the PDUFA VIII recommendations are finalized and implemented.

Sincerely,

A handwritten signature in black ink that reads "Randall L. Rutta". The signature is written in a cursive, flowing style.

Randall L. Rutta
Chief Executive Officer